The EveryLife Foundation asks Members of Congress to sign on to the Congressional Letter requesting the Food and Drug Administration (FDA) to fulfill the mandate to improve access to the Accelerated Approval pathway for rare diseases required by The Food and Drug Administration Safety and Innovation Act (FDASIA), [*P.L. 112-144*].

The EveryLife Foundation for Rare Diseases is dedicated to accelerating biotech innovation for rare disease treatments through science-driven public policy. We could do more with the science we already have and bring lifesaving treatments to millions of people with rare diseases.

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Background: The EveryLife Foundation joined more than 300 patient organizations in supporting the Faster Access to Specialized Therapies (FAST) Act, to amend the Federal Food, Drug, and Cosmetic Act with respect to Fast Track approval of certain orphan drugs. FAST sought to improve access to the Accelerated Approval process for rare diseases by empowering the FDA to use the best available science. FAST was included into FDASIA Section 901 and requires FDA to issue a draft guidance within one year of the bill being passed.

On June 25, FDA issued a draft guidance for Industry on Expedited Programs for Serious Conditions-Drugs and Biologics to fulfill the Section 901 mandate. Unfortunately, the draft guidance does not address the specific issues associated with rare disease drug development as clearly required by FDASIA.

Congress created the Fast Track process in 1992 to speed up the development of products intended to treat serious or life-threatening diseases with unmet medical needs. The Fast Track process permits expedited review under Accelerated Approval, which allows the use of surrogate endpoints (or biomarkers) to demonstrate the efficacy of a drug. Despite the success of Accelerated Approval in bringing new treatments for AIDS and cancer patients, there remain few approved treatments for rare diseases via the Accelerated Approval pathway even with the significant unmet need. The small number of patients with a particular rare disease makes it virtually impossible under the current FDA process to qualify novel surrogate endpoints since most rare diseases have never been treated before.

Solution: Congress must call on FDA to fulfill the requirements stated in FDASIA and improve access to the Accelerated Approval pathway for rare diseases.

Outcomes of Access to the Accelerated Approval Pathway for Rare Diseases:

- A more science-based and predictable FDA regulatory process
- A surge in investment and development activity for even the most rare disorders
- More patients with rare diseases will get earlier access to safe and effective treatments
- Increased investment in early-stage biotech companies focused on rare diseases will have a positive economic impact in local communities and bring new high paying biotechnology jobs

Supported by the following 58 Patient Organizations (as of September 6, 2013):

Abigail Alliance for Better Access to Developmental Drugs (VA)
Acid Maltase Deficiency Association (TX)
Addi & Cassi Fund (NV)
American Behcet's Disease Association (ABDA) (MI)
A-T Children's Project (FL)
Brian's Hope (CT)
Batten Disease Support & Research Association (BDSRA) (FL)
Celiac Sprue Association (NE)
Charcot-Marie-Tooth Association (IL)
Children's Cardiomyopathy Foundation (NJ)
Children's PKU Network (Phenylketonuria) (CA)
Children's Tumor Foundation (NY)
CARES Foundation, Inc. (NJ)
CureDuchenne (CA)
Drew's Hope Research Foundation (PA)
EveryLife Foundation for Rare Diseases (CA)
Fibromuscular Dysplasia Society of America (FMDSA) (OH)
Gene Spotlight Inc. (FL)
Global Genes | RARE Project (CA)
GT23 Foundation (NV)
Hannah's Hope Foundation (NY)
Hayden's Batten Disease Foundation Inc. (WI)
Huntington's Disease Society of America (NY)
I Have IIH (Idiopathic Intracranial Hypertension) (OH)
In Need of Diagnosis, Inc. (FL)
International Pemphigus & Pemphigoid Foundation (CA)
International Rett Syndrome Foundation (OH)
Idiopathic Pulmonary Hemosiderosis (IPH-NET) (MO)
International Scleroderma Network (MN)
Jonah's Just Begun (NY)
Kids With Heart National Association for Children's Heart Disorders, Inc. (WI)
Kortney Rose Foundation (NJ)
LAL Solace (Lysosomal Acid Deficiency) (AL)
Let Them Be Little X2 Foundation (NJ)
LGS Foundation (Lennox-Gastaut Syndrome) (NY)
Life Raft Group (NJ)
Lipodystrophy United (NM)
Little Miss Hannah Foundation (NV)
Mastocytosis Society (DC)
Metachromatic Leukodystrophy (MLD) Foundation (OR)
Multiple System Atrophy Coalition (NC)
National Gaucher Foundation (GA)
National Mucopolysaccharidosis Society (MPS) (NC)
National Tay-Sachs & Allied Diseases Association (MA)
Noah's Hope (IL)
NOMID Alliance (CA)
Parent Project Muscular Dystrophy (NJ)
Phelan-McDermid Syndrome Foundation (FL)
Ryan Foundation for Orhpan Disease Research (TX)
Sanfilippo Foundation for Children (TX)
Sarcoma Alliance (CA)
Sarcoma Foundation of America (MD)
Support for People with Oral and Head and Neck Cancer (SPOHNC) (NY)
Succinic Semialdehyde Dehydrogenase Deficiency (SSADH) (TX)
Taylor's Tale (NC)
Team Sanfilippo Foundation (NY)
United Pompe Foundation (CA)
Wilson Disease Association (WI)